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Chinese medicine drug for myofibrillar myopathy receives orphan drug designation by US FDA

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The Centre for Chinese Herbal Medicine Drug Development of Hong Kong Baptist University (HKBU) has developed a new drug using effective components of a Chinese herbal medicine, Chaenomelis Fructus, for the treatment of the rare disease, myofibrillar myopathy. The drug has obtained the orphan drug designation from the US Food and Drug Administration (FDA), and is the first botanical drug in Hong Kong to receive this qualification.

Success in obtaining orphan drug (a drug used for treating rare disease) designation will accelerate the approval process of the new drug, including speeding up of the review process, waiver of the marketing authorisation fee, and seven years of market exclusivity for the approved product. The research team plans to submit an Investigational New Drug application to FDA in two years to conduct clinical trials.

Myofibrillar myopathy, primarily caused by genetic mutations, including the BAG3 gene, is a rare hereditary neuromuscular disorder with symptoms resembling muscular dystrophy. It typically manifests during adolescence and is characterised by severe clinical symptoms, including progressive muscle weakness, muscle atrophy, motor impairment, muscle stiffness, respiratory muscle involvement, and cardiomyopathy.

Currently, there is no effective treatment for myofibrillar myopathy, and the high cost of treatment poses significant burdens for patients, their families and society. Due to the rarity of this disease, researchers are still exploring the treatment approaches for this disease and evaluating their safety and efficacy.